Our STN: BL 125771/0

LATE-CYCLE MEETING MEMORANDUM

Bioverativ Therapeutics Inc.
Attention: Hei-Jen Sun, PhD
55 Corporate Drive
Bridgewater, NJ 08807

Dear Dr. Sun:

Attached is a copy of the memorandum summarizing your December 19, 2022, Late-Cycle Meeting Teleconference with CBER. This memorandum constitutes the official record of the Teleconference. If your understanding of the Teleconference outcomes differs from those expressed in this summary, it is your responsibility to communicate with CBER in writing as soon as possible.

Please include a reference to the appropriate Submission Tracking Number (STN) in future submissions related to the subject product.

If you have any questions, please contact Niloofar Kennedy at Niloofar.kennedy@gmail.com.

Sincerely,

Basil Golding, MD
Director
Division of Plasma Protein Therapeutics
Office of Tissues and Advanced Therapies
Center for Biologics Evaluation and Research

Late-Cycle Meeting Summary

Meeting Date and Time: December 19, 2022, 10:00-11:00 am, ET
Meeting Location: Zoom Teleconference
Application Number: BLA 125771/0
Product Name:
Antihemophilic Factor (Recombinant), Fc-VWF-XTEN Fusion Protein
Proposed Indications:
Treatment of adults and children with Hemophilia A (congenital Factor VIII deficiency) for: (1) Routine prophylaxis to reduce the frequency of bleeding episodes; (2) On-demand treatment and control of bleeding episodes; and (3) Perioperative management of bleeding.

Applicant Name:
Bioverativ Therapeutics, Inc.

Meeting Chair: Zuben Sauna, PhD
RPMs: Niloofar Kennedy

FDA ATTENDEES
Adnan Jaigirdar, MD, FACS, CBER/OTAT/DCEPT
Basil Golding, MD, CBER/OTAT/DPPT
Carolyn Renshaw, CBER/OCBQ/DMPQ
Daniel Lagasse, PhD, CBER/OTAT/DPPT
Denise Gavin, PhD, CBER/OTAT/DCGT
Dennis Cato, CBER/OCBQ/DIS/BMB
Donald Ertel, PhD, CBER/OCBQ/DMPQ
Gregory Price, PhD, CBER/OCBQ/DMPQ
Ileana Marrero-Berrios, PhD, CBER/OTAT/DCGT
Jiang Hu, PhD, CBER/OBPV
Kristine Khuc, PharmD, CBER, OCBQ, DCM, APLB
Lihan Yan, PhD, CBER/OBPV/DB/TEB2
Lin Huo, PhD, CBER/OBPV/DB
Mary Rubin, PhD, CBER/OBPV
Maureen DeMar, CBER/OCBQ/DMPQ
Niloofar Kennedy CBER/OTAT/DRPM
Parmesh Dutt, PhD, CBER/OCBQ/DBSQC
Ramani Sista, PhD, CBER/OTAT/DRPM
Tejashri Purohit-Sheth, MD, CBER/OTAT/DCEPT
Tyree Newman, MDiv, CBER/OTAT/DRPM
Ye Xiong, CDER/OTS
Zuben Sauna, PhD, CBER/OTAT/DPPT/HB

Rachael Anatol, PhD, CBER/OTAT
Wilson W. Bryan, MD, CBER/OTAT

APPLICANT ATTENDEES

Craig Benson, MD Senior Global Project Head-Hemophilia
Annemieke Willemze, MD, PhD Global Project Head, Rare Diseases & Rare Blood Disorders
Ekta Seth Chhabra, PhD Sr Clinical Research Director, Rare Diseases & Rare Blood Disorders
Suresh Katragadda, PhD Director, Clinical Pharmacology
Abhimanyu Yarramaneni, MD Global Safety Officer, Pharmacovigilance
Zhiying Qiu, PhD Statistical Project Leader, Biostatistics and programming
Paragi Patel, PharmD Associate Director, Global Regulatory Affairs Labeling
Alice Sebastian, Ph.D Senior Manager, Global CMC Regulatory Affairs
HeiJen Sun, PhD Director, Global Regulatory Affairs
Priti Lad, PharmD Senior Director, Global Regulatory Affairs – US
Kayla Buckstein Manager, Global Regulatory Affairs – US

BACKGROUND

BLA 125771/0 was submitted on June 30, 2022, for Antihemophilic Factor (Recombinant), Fc-VWF-XTEN Fusion Protein (ALTUVIIIO).

Proposed indication:
Treatment of adults and children with Hemophilia A (congenital Factor VIII deficiency) for:

1. Routine prophylaxis to reduce the frequency of bleeding episodes;
2. On-demand treatment and control of bleeding episodes;
3. Perioperative management of bleeding

PDUFA goal date: February 28, 2023

In preparation for this meeting, FDA issued the Late-Cycle Meeting Materials on December 13, 2022.

DISCUSSION

1. Discussion of Substantive Review Issues

    a. No significant issues/major deficiencies identified at this time.

2. Information Requests

    a. No major safety concerns have been identified to date.

3. Risk Management Actions (e.g., REMS)

    a. At this time, the review teams have not identified a need for a Risk Evaluation Mitigation Strategy (REMS). The Risk Management materials are under review and any comments will be sent in an IR.

4. Postmarketing Requirements/Postmarketing Commitments

    a. There are no PMR/PMCs anticipated at this time.

5. Major Labeling Issues

    a. There are no major labeling issues at this time.

6. Review Plans

    a. Currently there is no plan to extend the PDUFA goal date.

7. Applicant Questions

    a. We understand that the FDA will send the clinical information request this week. Is it possible to send today or tomorrow so we can provide the response within this week? FDA confirmed that the Clinical IR will be sent on December 19, 2022.

    b. FDA will follow-up with a response via email to the Sponsor's request for a review of syringe label, vial label and carton earlier than January 27 2023.

8. Wrap-up and Action Items

    a. This application has not yet been fully reviewed by the signatory authorities, Division Directors and Review Committee Chair and therefore, this meeting did not address the final regulatory decision for the application.